Reference number(s)
1927-A

SPECIALTY GUIDELINE MANAGEMENT

NPLATE (romiplostim)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

A. FDA-Approved Indications
Nplate is indicated for the treatment of thrombocytopenia in:
1. Adult patients with immune thrombocytopenia (ITP) who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy.
2. Pediatric patients 1 year of age and older with ITP for at least 6 months who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy.

B. Compendial Uses
1. Myelodysplastic syndromes, for lower risk disease in patients with severe or refractory thrombocytopenia following disease progression or no response to hypomethylating agents or immunosuppressive therapy
2. Severe thrombocytopenia post cancer chemotherapy

All other indications are considered experimental/investigational and not medically necessary.

II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:
A. Immune thrombocytopenia: pretreatment and current platelet counts
B. Severe thrombocytopenia post cancer chemotherapy: pretreatment and current platelet counts

III. EXCLUSIONS

Coverage will not be provided for members with the following exclusion: concomitant use of Nplate with other thrombopoietin receptor agonists (e.g., Promacta, Doptelet, Mulpleta) or with spleen tyrosine kinase inhibitors (e.g., Tavalisse)

IV. CRITERIA FOR INITIAL APPROVAL

A. Immune thrombocytopenia (ITP)
Authorization of 6 months may be granted for treatment of ITP when both of the following criteria are met:
1. Inadequate response or intolerance to prior therapy with corticosteroids, immunoglobulins, or splenectomy.

2. Untransfused platelet count at time of diagnosis is less than $30x10^9$/L OR $30x10^9$/L to $50x10^9$/L with symptomatic bleeding (e.g., significant mucous membrane bleeding, gastrointestinal bleeding or trauma) or risk factors for bleeding (see Section VI).

B. Myelodysplastic Syndromes

Authorization of 12 months may be granted for treatment of myelodysplastic syndromes when both of the following criteria are met:

1. Member has lower risk disease defined as Revised International Prognostic Scoring System (IPSS-R) (Very Low, Low, Intermediate), International Prognostic Scoring System (IPSS) (Low/Intermediate-1), WHO classification-based Prognostic Scoring System (WPSS) (Very Low, Low, Intermediate).
2. Member has severe or refractory thrombocytopenia following disease progression or no response to hypomethylating agents (such as azacitidine and decitabine) or immunosuppressive therapy.

C. Severe thrombocytopenia post cancer chemotherapy

Authorization of 6 months may be granted for treatment of severe thrombocytopenia post cancer chemotherapy when the platelet count is less than $50x10^9$/L.

V. CONTINUATION OF THERAPY

A. Immune thrombocytopenia (ITP)

1. Authorization of 3 months may be granted to members with current platelet count less than $50x10^9$/L for whom the platelet count is not sufficient to prevent clinically important bleeding and who have not received a maximal Nplate dose for at least 4 weeks.
2. Authorization of 12 months may be granted to members with current platelet count less than $50x10^9$/L for whom the current platelet count is sufficient to prevent clinically important bleeding.
3. Authorization of 12 months may be granted to members with current platelet count of $50x10^9$/L to $200x10^9$/L.
4. Authorization of 12 months may be granted to members with current platelet count greater than $200x10^9$/L to less than or equal to $400x10^9$/L for whom Nplate dosing will be adjusted to achieve a platelet count sufficient to avoid clinically important bleeding.

B. Myelodysplastic Syndromes

Authorization of 12 months may be granted for continued treatment of myelodysplastic syndromes in members who experience benefit from therapy (e.g., increased platelet counts, decreased bleeding events, reduced need for platelet transfusions).

C. Severe thrombocytopenia post cancer chemotherapy

Authorization of 6 months may be granted for continued treatment of severe thrombocytopenia post cancer chemotherapy in members who experience benefit from therapy (e.g., increased platelet counts, decreased bleeding events, reduced need for platelet transfusions) and the platelet count remains less than $100x10^9$/L.

VI. APPENDIX

<u>Examples of risk factors for bleeding (not all inclusive)</u>

- Undergoing a medical or dental procedure where blood loss is anticipated
- Comorbidity (e.g., peptic ulcer disease, hypertension)
- Mandated anticoagulation therapy

- Profession (e.g., construction worker) or lifestyle (e.g., plays contact sports) that predisposes patient to trauma

VII. REFERENCES

1. Nplate [package insert]. Thousand Oaks, CA: Amgen Inc.; October 2019.
2. The NCCN Drugs & Biologics Compendium® © 2019 National Comprehensive Cancer Network, Inc. https://www.nccn.org. Accessed June 13, 2019.
3. The NCCN Clinical Practice Guidelines in Oncology® Myelodysplastic Syndrome (Version 2.2019). © 2019 National Comprehensive Cancer Network, Inc. https://www.nccn.org. Accessed June 13, 2019.
4. Bose P, Hussein KK, Terrell DR, et al. Successful treatment of cyclic thrombocytopenia with thrombopoietin-mimetic agents: a report of two patients. *Am J Hematol*. 2009;84:459-461.
5. Rice L, Nichol JL, McMillan R, et al. Cyclic immune thrombocytopenia responsive to thrombopoietic growth factor therapy. *Am J Hematol*. 2001;68:210-214.
6. Neunert C, Lim W, Crowther M, et al. The American Society of Hematology 2011 evidence-based practice guideline for immune thrombocytopenia. *Blood.* 2011;117(16):4190-4207.
7. Provan D, Stasi R, Newland AC, et al. International consensus report on the investigation and management of primary immune thrombocytopenia. *Blood.* 2010;115(2):168-186.
8. Rodeghiero F, Stasi R, Gernsheimer T, et al. Standardization of terminology, definitions and outcome criteria in immune thrombocytopenic purpura of adults and children: report from an international working group. *Blood*. 2009;113(11):2386-2393.
9. NCCN hematopoietic growth factors. Short-term recommendations specific to issues with COVID-19 (SARS-CoV-2). National Comprehensive Cancer Network, Inc. Available at: https://www.nccn.org/covid-19/pdf/HGF_COVID-19.pdf. Accessed April 16, 2020.